

The EFGCP Children's Medicines Working
Party 3rd Annual Conference

*EU Paediatric Regulation: First European
Experiences & Strategic Outlook*

8-9 October 2007 - Management Centre Europe, Brussels, Belgium

Meeting report



'where science & ethics meet'

A fresh start for children?

EFGCP News editor Peter Wrobel reports on discussion and debate about the workings of the new Paediatric Regulation. Is it a fresh start for children – or simply too soon to tell?

It was a long time coming, but the European Union's Paediatric Regulation finally came into force at the start of the year. And by the end of July its key instrument – the Paediatric Committee – was open for business. So, has a new era finally dawned for children and their treatment?

That was the background for the annual conference of the EFGCP Children's Working party – its third – which took place in Brussels in October to review progress so far. And the verdict? Although in many areas it's still too soon to tell, the outlook is generally positive. As Frank Wells, co-chair of the EFGCP Ethics Working Party, put it: "Yes, it's early days, but my goodness we are making progress."

Almost 100 delegates met for an intensive day of talks, workshops and discussion, centred around two sessions: First Experiences, and Global Thoughts on Strategic Research.

Experience limited

The first experiences were on the basis of some fairly limited data – perhaps more limited than might have been anticipated. The figures were spelled out by Daniel Basseur, the new Chair of the Paediatrics Committee, and Agnes Saint-Raymond, Head of Sector for Scientific Advice and Orphan Drugs at the European Medicines Evaluation Agency in London.

As Basseur explained, "The core of the business of the committee is the assessment of Paediatric Investigation Plans (PIPs)." So far, the committee has received 41 Paediatric Investigation Plans (PIPs), of which 29 related to products already under patent, only 11 for products not yet authorised, and just 1 for an off-patent medicine being developed for use in children.

So experience is not yet broad. And, of course, given the timescales involved, there were no final decisions yet.

For its part, industry is highly positive, but it is still getting to grips with the new procedures. As Liz Carter, from Pfizer, said, "For all of us the processes are new, exciting, but challenging." In particular, industry would like to be in more direct communication with the rapporteurs and peer reviewers who play a central role in the assessment of PIPs.

Klaus Rose, head paediatrics at Roche and chairman of the EFGCP Children's Medicines Working Party, noted that PIP applications for early-stage oncology drugs were only just starting to come, and there were many unknowns. Hitherto, much research had been done by academic collaborative groups. Now companies were getting involved. But, he said, "If companies are expected to move into a ground that until now has been covered by collaborative groups, where will EMEA draw the line for early clinical PIPs if the results are negative?" Those and other issues, said Rose, will need more discussion, starting with the joint EMEA/ EFGCP/DIA workshop on paediatric oncology in London on 15 November.

In addition, Rose questioned whether other conditions, such as asthma, rheumatoid arthritis and obesity, were actually the same illnesses in adults as in children.

As expected, the break-out sessions provided fertile ground for discussion. From the workshop on First Experiences with the EMEA came queries about how a PIP should be amended – and that question in itself led to questions about what a PIP actually was: a concept, or a detailed plan? The EMEA, it was felt, should put up one or more examples of PIPs on its website as a guide to others.

Though, it is still too early to talk about deficiencies in the process and in the Paediatric Committee, one topic was already identified: "In a word, transparency," said rapporteur Tsveta Schyns from the European

Network for Research on Alternating Hemiplegia. "It is absolutely needed. Dialogue with industry and with patients."

Industry wants dialogue

Over in the second workshop, industry's experiences were under the spotlight. The need for dialogue was emphasised again: "These are early days," said rapporteur Melissa Tassinari from Pfizer. "Maintain the dialogue. We will be in a much better space this time next year." Among the various lessons learned, two stood out: about PIPs for early-stage research, and the need for a global approach.

It seems that there was too much detail in the early PIP applications. The advice to companies from the workshop was just to include enough information to deliver your thinking on your paediatric programme so that discussions can begin – but to step back from filling out everything in the template. "If you don't have an answer, that's the answer." And companies wanted the US FDA involved early on. The goal is a single paediatric plan.

From the workshop looking at the issue from the point of view of paediatric research networks came a clear message: "We need better research. The area is in its infancy and a major role is to adopt common standards and working procedures, particularly in relation to databases," said rapporteur Fergal Donnelly from the EU Commission.

In the second half of the conference, delegates heard from Mark Del Monte from the American Academy of Pediatrics about the battle to renew and update the legislation there, from Hidefumi Nakamura, from Japan's National Centre for Child Health and Development, about much-needed plans to stimulate paediatric research in Japan, and from Sam Maldonado, vice president of paediatric drug development for Johnson & Johnson.

Avoid duplication

"We would like to have a single global paediatric programme to avoid duplication and redundancy, and avoid unnecessary exposure of children to research," said Maldonado. After all, he said, although the practice of medicine may differ between the US and the EU, the underlying principles are the same.

The first of the three concurrent workshops on strategies for global research concentrated on the role of research networks – were national ones better than disease-specific ones? – and concluded that you needed both, in partnership. The second workshop focused on clinical trial design

The final workshop looked at the impact of the European Union's Clinical Trials Directive on paediatric clinical research – and concluded that the impact was flawed because the Directive meant different things in different countries. Turn it into a Regulation that all countries had to follow, said delegates, and what's more, have a central European Ethics Committee to approve multinational trials.

For further information:

Klaus Rose, Chairman, EFGCP Children's Medicines Working Party (klaus.rose@efgcp.be)

*EFGCP Secretariat
Square de Meeüs – Rue de l'Industrie 4
BE-1000 Brussels, Belgium
Tel: +32.2.732 87 83
Fax: +32.2.503 31 08
E-mail: secretariat@efgcp.be
Website: www.efgcp.be*